



PTO/SB/08b (08-03)

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Substitute for form 1449A/PTO		Complete if Known	
<b>INFORMATION DISCLOSURE STATEMENT BY APPLICANT</b>  (use as many sheets as necessary)		Application Number	10/552,287
		Filing Date	April 18, 2004
		First Named Inventor	Anthony FUTERMAN et al
		Group Art Unit	1656
		Examiner Name	Not Yet Assigned
Sheet	1	Of	4
Attorney Docket Number 30227			
<b>OTHER PRIOR ART - NON PATENT LITERATURE DOCUMENTS</b>			
Examiner Initials	Cite No. <sup>1</sup>	Include name of the author (in CAPITAL LETTERS), title of the article (when appropriate), title of the item (book, magazine, journal, serial symposium, catalog, etc.) date, page(s), volume-issu number(s), publisher, city and/or country where published.	T <sup>2</sup>
	1	Grabowski et al. "Human Acid $\beta$ -Glucosidase - Use of Conduirito B Epoxide Derivatives to Investigate the Catalytically Active Normal and Gaucher Disease Enzymes", The Journal of Biological Chemistry, 261(18): 8263-8269, 1986.	
	2	Berg-Fussman et al. "Human Acid $\beta$ -Glucosidase - N. Glycosylation Site Occupancy and the Effect of Glycosylation on Enzymatic Activity", The Journal of Biological Chemistry, 268(20):14861-14866, 1993.	
	3	Roeber et al. "Crystallization and Preliminary X-Ray Analysis of Recombinant Human Acid Beta-Glucocerebrosidase, A Treatment for Gaucher's Disease", Biological Crystallography, D59: 343-344, 2003.	
	4	Dvir et al. "X-Ray Structure of Human Acid- $\beta$ -Glucosidase, the Defective Enzyme in Gaucher Disease", The EMBO Journal, P.1-27, 2003.	
	5	Sawkar et al. "Chemical Chaperones Increase the Cellular Activity of N370S $\beta$ -Glucosidase, A Therapeutic Strategy for Gaucher Disease", PNAS, 99(24): 15428-15433, 2002.	
	6	Erickson et al. "Biosynthesis of the Ltsomal Enzyme Glucocerebrosidase", The Journal of Biological Chemistry, 260(26): 14319-14324, 1985.	
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	9	Amaral et al. "Type 1 Gaucher Disease: Identification of N396T and Prevalence of Glucocerebrosidase Mutations in the Portuguese", Human Mutation, 8: 280-281, 1996.	
	10	Beutler "Economic Malpractice in the Treatment of Gaucher's Disease", The American Journal of Medicine, 97: 1-2, 1994.	
	11	Beutler et al. "Gaucher Disease", The Metabolic and Molecular Bases of Inherited Disease, Chap.146: 3635-3668, 2001.	
	12	Beutler et al. "Two New Gaucher Disease Mutations", Human Genetics, 93: 209-210, 1994.	
	13	Brünger et al. "Crystallography & NMR System: A New Software Suite for Macromolecular Structure Determination", Acta Crystallographica Section D, 54: 905-921, 1998.	
	14	Buccoliero et al. "The Role of Sphingolipids in Neural Development: Lessons From Models of Sphingolipid Storage Diseases", Neurochemical Research, 27(7/8): 565-574, 2002.	
Signature		/David Steadman/	Considered
			06/12/2006

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<b>OTHER PRIOR ART - NON PATENT LITERATURE DOCUMENTS</b>		
15	Charrow et al. "The Gaucher Registry. Demographics and Disease Characteristics of 1698 Patients With Gaucher Disease", Archive of Internal Medicine, 160: 2835-2843, 2000.	
16	Chi et al. "Crystal Structure of the $\beta$ -Glucosidase From the Hyperthermophile Thermosphaera Aggregans: Insights Into Its Activity and Thermostability", FEBS Letters, 445: 375-383, 1999.	
17	Cox et al. "Novel Oral Treatment of Gaucher's Disease With N-Butyldoxynojirimycin (OGT 918) to Decrease Substrate Biosynthesis", The Lancet, 355: 1481-1485, 2000.	
18	Davies et al. "Structures and Mechanisms of Glycosyl Hydrolases", Structure, 3: 853-859, 1995.	
19	Dinur et al. "Human Acid $\beta$ -Glucosidase: Isolation and Amino Acid Sequence of A Peptide Containing the Catalytic Site", Proc. Natl. Acad. Sci. USA, 83: 1660-1664, 1986.	
20	Fabrega et al. "Site Actif de la Glucocérébrosidase Humaine: Prédiction Structurale et Validation Expérimentale", Journal de la Société de Biologie, 196(2): 151-160, 2002. Article in French.	
21	Fabrega et al. "Human Glucocerebrosidase: Heterologous Expression of Active Site Mutants in Murine Null Cells", Glycobiology, 10(11): 1217-1224, 2000.	
22	Fan "A Contradictory Treatment for Lysosomal Storage Disorders: Inhibitors Enhance Mutant Enzyme Activity", Trends in Pharmacological Sciences, 24(7): 355-360, 2003.	
23	De La Fortelle et al. "Maximum-Likelihood Heavy-Atom Parameter Refinement for Multiple Isomorphous Replacement and Multiwavelength Anomalous Diffraction Methods", Methods in Enzymology, 276: 472-494, 1997.	
24	Futerman et al. "The Cell Biology of Lysosomal Storage Disorders", Nature Reviews in Molecular & Cellular Biology, 5: 554-565, 2004.	
25	Futerman et al. "New Directions in the Treatment of Gaucher Disease", Trends in Pharmacological Sciences, 25(3): 147-151, 2004.	
26	Grabowski et al. "Enzyme Therapy for Lysosomal Storage Disease: Principles, Practice, and Prospects", Annual Reviews in Genomics & Human Genetics, 4: 403-436, 2003.	
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28	Grabowski et al. "Acid $\beta$ -Glucosidase: Enzymology and Molecular Biology of Gaucher Disease", Critical Reviews in Biochemistry and Molecular Biology, 25(6): 385-414, 1990.	
29	Grace et al. "Analysis of Human Acid $\beta$ -Glucosidase by Site-Directed Mutagenesis and Heterologous Expression", The Journal of Biological Chemistry, 269(3): 2283-2291, 1994.	

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30	Henrissat et al. "New Families in the Classification of Glycosyl Hydrolases Based on Amino Acid Sequence Similarities", Biochemical Journal, 293: 781-788, 1993.		
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39	Legler "Glycoside Hydrolases: Mechanistic Information From Studies With Reversible and Irreversible Inhibitors", Advances in Carbohydrate Chemistry and Biochemistry, 48: 319-384, 1990.		
40	Lloyd-Evans et al. "Glucosylceramide and Glucosylsphingosine Modulate Calcium Mobilization From Brain Microsomes Via Different Mechanisms", The Journal of Biological Chemistry, 278(26): 23594-23599, 2003.		
41	Meivar-Levy et al. "Analysis of Glucocerebrosidase Activity Using N-(1-[14C]Hexanoyl)-D-Erythro-Glucosylsphingosine Demonstrates A Correlation Between Levels of Residual Enzyme Activity and the Type of Gaucher Disease", Biochemical Journal, 303: 377-382, 1994.		
42	Miao et al. "Identification of Glu340 as the Active-Site Nucleophile in Human Glucocerebrosidase by Use of Electrospray Tandem Mass Spectrometry", The Journal of Biological Chemistry, 269(15): 10975-10978, 1994.		
43	Mistry et al. "Therapeutic Delivery of Proteins to Macrophages: Implications for Treatment of Gaucher's Disease", The Lancet, 348: 1555-1559, 1996.		
44	Morel et al. "Effect of Mutations Within the Peripheral Anionic Site on the Stability of Acetylcholinesterase", Molecular Pharmacology, 55: 982-992, 1999.		

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Sheet	4	Of	
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## OTHER PRIOR ART – NON PATENT LITERATURE DOCUMENTS

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|----|---|
| 45 | Murshudov et al. "Efficient Anisotropic Refinement of Macromolecular Structures Using FFT", <i>Acta Crystallographica Section D</i> , 55: 247-255, 1999.  |
| 46 | Nyholm et al. "The Effect of Hydrogen Bonds on the Conformation of Glycosphingolipids. Methylated and Unmethylated Cerebroside Studied by X-Ray Single Crystal Analysis and Model Calculations", <i>Chemistry and Physics of Lipids</i> , 52: 1-10, 1990. |
| 47 | Otwinowski et al. "Processing of X-Ray Diffraction Data Collected in Oscillation Mode", <i>Methods in Enzymology</i> , 276(Chap.20): 307-326, 1997.   |
| 48 | Perrakis et al. "Automated Protein Model Building Combined With Iterative Structure Refinement", <i>Nature Structural Biology</i> , 6(5): 458-463, 1999.  |
| 49 | Qasba et al. "Substrate-Induced Conformational Changes in Glycosyltransferases", <i>Trends in Biochemical Sciences</i> , 30(1): 53-62, 2005.  |
| 50 | Schüttelkopf et al. "PRODRG: A Tool for High-Throughput Crystallography of Protein-Ligand Complexes", <i>Acta Crystallographica Section D</i> , 60: 1355-1363, 2004.  |
| 51 | Usón et al. "Advances in Direct Methods for Protein Crystallography", <i>Current Opinion in Structural Biology</i> , 9: 643-648, 1999.  |
| 52 | Weinreb et al. "Effectiveness of Enzyme Replacement Therapy in 1028 Patients With Type 1 Gaucher Disease After 2 to 5 Years of Treatment: A Report From the Gaucher Registry", <i>American Journal of Medicine</i> , 113: 112-119, 2002.                  |
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| 54 | Wilkening et al. "Lysosomal Degradation on Vesicular Membrane Surfaces", <i>The Journal of Biological Chemistry</i> , 273(46): 30271-30278, 1998.   |
| 55 | Kabsch, "Automatic Processing of Rotation Diffraction Data From Crystals of Initially Unknown Symmetry and Cell Constants", <i>Journal of Applied Crystallography</i> , 26: 795-800, 1993.  |

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